



Network of Rare Blood Disorder  
Organizations  
Réseau des Associations Vouées  
aux Troubles Sanguins Rares

## **Comprehensive Care Conference**

**Toronto | November 18, 2017**

Sheraton Toronto Airport Hotel and Conference Centre, 801 Dixon Rd Toronto

### **SUMMARY NOTES**

#### **EXECUTIVE SUMMARY**

This unique conference brought together patients, physicians, pharma, politicians, and policy makers to learn and share perspectives on the value of investments in Comprehensive Care.

When it comes to rare blood disorders, finding the balance between optimal patient care and efficient stewardship of healthcare dollars is a challenge felt by doctors, hospital administrators, and government officials alike.

This conference was designed to present a growing body of evidence from both domestic and international clinics where innovative strategies are:

- improving patient and caregiver experiences and outcomes
- improving safety through tracking of product use
- increasing cost effectiveness
- resulting in shorter and fewer hospital stays
- reducing product waste

#### **Throughout the day, several common themes emerged:**

- There is a very compelling argument for investment in Comprehensive Care:
  - It is the right thing to do, as it results in improved outcomes for patients, and
  - It is the smart thing to do, as it improves efficiencies and saves costly waste on product.
- The evidence needs to be supported by patient story, and tailored to highlight how it aligns with the goals of each specific audience.
- There is a strong leadership role for patient groups to play, in advocating, sharing stories, and collecting data.
- The importance of sustainable funding was apparent in each care model presented.
- Collaboration in advocating for Comprehensive Care will produce the best results.



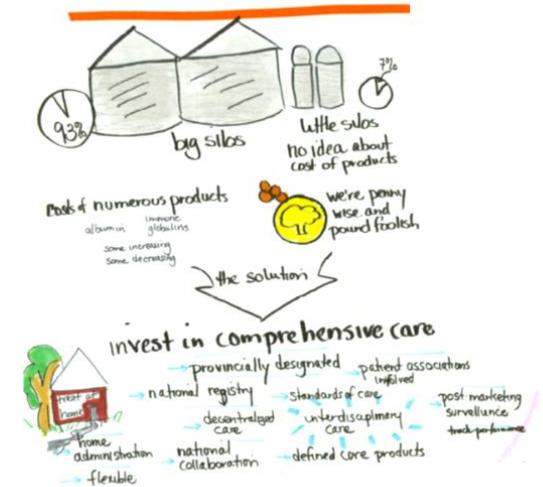
PROCEEDINGS

**Why Comprehensive Care?**

David Page, CHS/NRBDO

[\(slide deck\)](#)

Mr. Page presented the blood product budget as the large silo making up 93% of the cost for those receiving blood products, and the care delivery budget as the small silo making up only 7% of the total cost. These 2 systems operate with very little understanding or interest in each other. It is only by looking at both holistically that we can understand how relatively small investments in care delivery can have positive impacts on the overall budget, while also providing much improved care for patients.



Mr. Page outlined the 11 pillars of comprehensive care, as determined by consensus at the 2006 NRBDO Conference and shared an update on the progress made across Canada by the NRBDO member patient organizations, concluding that there is still a lot of room for improvement.

**Evidence to Support Comprehensive Care/Integrated Care Delivery Model**

National Hemophilia Foundation: McMaster Guideline on Care Models for Haemophilia Management – Dr. Menaka Pai

[\(slide deck\)](#)



Dr. Pai's presentation described the challenges of guideline creation in rare diseases, shared how novel guideline creation methods were used to develop an evidence-based guideline on hemophilia care, and highlighted the NHF-McMaster Guidelines recommendations around models of care.

- Guidelines help us make good decisions, understanding the evidence behind the choices and the impact of our choices, which can then be customized to a patient and their family.
- Rare diseases pose a unique challenge to guideline methodologists. We need to use rigorous, transparent, and creative strategies to create guidelines that improve patient care: extrapolate from more common diseases, interview key stakeholder groups, access “expert evidence” in a systematic way.
- When creating the guidelines for hemophilia care, the researchers compared Integrated (Comprehensive) care settings, Specialists in a non-specialized setting, and non-specialists in a non-specialized setting, with the goal of identifying best practices in hemophilia care and delivery and optimize hemophilia care for each patient. Dr. Pai shared the process followed in detail. High quality evidence was scarce, so they completed parallel searches for other chronic diseases and extrapolated evidence to inform the recommendations for hemophilia, and also interviewed hemophilia patients.
- The final recommendation was that for people with hemophilia, the integrated care model should be used over non-integrated care models, as it was found to be beneficial to patients and also cost-effective. They recommended that a care team should include: a hematologist, specialized hemophilia nurse, physical therapist, social worker, and round-the-clock access to a specialized coagulation laboratory.
- Dr. Pai's recommendation to other treatment centres was to build data collection and analysis capacity, so they could conduct high quality, well-organized studies. Patient groups can also contribute with quality patient surveys and data collection, to help build a stronger base of evidence for the care that will benefit them the most.



## Successful Models of Comprehensive Care – Highlighting the Bright Spots in Canada



### Saskatchewan Bleeding Disorders Program – Dr. Kelsey Brose [\(slide deck\)](#)

Dr. Kelsey Brose presented on the Saskatchewan Bleeding Disorders Program, describing how the program was formed and some of their successes and challenges.

Dr. Brose oversees the bleeding disorders clinic, staffed with physicians, nurses, physiotherapist, social worker, and administrators. They also work closely with a genetics counsellor, OB/GYN, a dentist, and a pain consultant. They are able to offer ambulance services, ultrasound on site, reimbursed parking, and emergency funding.

It is a true provincial program and they serve all the bleeding disorder patients in Saskatchewan. Due to the challenge of patients sometimes having to drive up to 6 hours to the Saskatoon clinic, they now have 2 additional sites: Regina and Prince Albert.

The proposal for the program was submitted to the Ministry of Health in 1999, with strong advocacy by Hemophilia Saskatchewan. It was accepted in 2000 and direct provincial funding was officially legislated, with the first clinic opening in 2001. While Dr. Brose outlined some challenges, he credits a close working relationship with the patient advocacy organization and the provincial ministry, along with regular strategic planning, for the success of the program.

**John Akabutu Comprehensive Care Centre for Bleeding Disorders** – Dr. Bruce Ritchie  
[\*\(slide deck\)\*](#)

Dr. Bruce Ritchie oversees a comprehensive care clinic for numerous blood disorders in Edmonton, Alberta. They offer comprehensive care and home treatment programs for bleeding disorders, HAE, Immunodeficiency, Hemoglobinopathy, Porphyrin, Ehlers Danlos syndrome, and Gaucher disease.

Dr. Ritchie shared data on the cost-effectiveness of home treatment vs hospital-based treatment, noting not just the decrease in labour costs associated with care but also the significant increased capacity. Important to consider as well is the quality of life improvement for patients who can self-infuse at home.

**Successful Models of Comprehensive Care - International Models**

**HAE Care in Italy** – Dr. Marco Cicardi  
[\*\(slide deck\)\*](#)

Dr. Cicardi presented on Angioedema in some detail, describing the different types and genetic mutations. Approaches to the treatment of HAE include on demand (at the time of an attack), short term prophylaxis (when patients are at risk), and long-term prophylaxis (given continuously to prevent appearance of symptoms).

Dr. Cicardi shared about their approach to self-administration of treatment in Italy, and the burden of disease for HAE, along with the studies done in Europe to better understand the management and impact of HAE from the patient perspective.

ITACA is the Italian Network for C1-INH-HAE. It is a multidisciplinary panel of experts together with the Italian HAE Patient Association. They have established standards that need to be met to qualify as an HAE reference centre (there are 18 reference centres), updated the Italian guidelines for the diagnosis and treatment of C1-INH-HAE, and created a database of C1-INH-HAE patients. There is an established “regional HAE diagnostic and therapeutic path” in Italy. With these measures in place the percentage of treated attacks has risen by over 60%.

The HAE registry is a tool for monitoring patients, monitoring quality of care, and expanding knowledge. Dr. Cicardi believes it helps them take a holistic approach to HAE care.

**HAE Care in the USA** – Dr. Marc Riedl  
[\*\(slide deck\)\*](#)

Dr. Riedl is the Clinical Director of the US HAEA Angioedema Center at the University of California, San Diego. This is the national centre of excellence for HAE treatment, but it is funded by private donations and industry sponsorship, not federal or state health departments.

Dr. Riedl shared how the treatment plans and medical management plans were developed for HAE. They had found that there were less than 250 physicians treating HAE in the US, and that 70% of US patients were managed by a physician who had experience with less than 10 HAE patients. There was also inconsistency with consensus guidelines, and only half of first-degree relatives were being screened.

With expert physician involvement, recommendations were published in 2013. The recommendation included national referral centres or networks. The US HAEA Angioedema Centre of UC San Diego was established as a collaborative effort. Their model is patient-centred, with expert and experienced staff, full range of treatment options, a research component, and clinical and lab resources. They develop individualized HAE treatment plans, and the US HAEA, the national patient organization, plays a large role in providing resources and education.

The centre sees 95% of their patients from 35 different states, with the other 5% coming from Canada, Mexico, Central and South America, Asia and Africa. They also serve as the hub of the US HAEA Research Network, and house the US HAEA patient registry.

They continue to work to improve HAE management through clinical care, research, and education efforts. Evidence gathered shows that improved HAE Care has led to a decrease in acute attack treatments in the hospitals, urgent care clinics, and emergency departments as patients are able to treat themselves at home. Other success factors include improved diagnostic accuracy, access to effective medication, rational use of HAE-specific drugs, and high patient satisfaction scores.

### **International Models of Care for PID – Johan Prevot (via video)** [\(slide deck\)](#)

Johan Prevot is the Executive Director of IPOPI, the international patient organization for primary immunodeficiencies. He presented an introduction on IPOPI, the PID Principles of Care, PID comprehensive care models in France and EU-funded projects, and the role of the patient organizations. There are over 300 PIDs defined in 9 different groups and it is estimated that 80% of patients globally do not have access to adequate care.

The PID Principles of Care were published as a call to action in 2015, calling for specialized centres, registries, international research collaborations, a role for patient organizations, management and treatment options, and managing diagnosis and care in all countries.

Comprehensive Care for PID is from fetus to old age, and includes diagnostics, individualized patient approaches, diverse treatment options, multidisciplinary holistic care, and patient and family support.

The highest number of registered patients in the EU are at the France national reference centre for PID (CEREDIH). This centre was created in 2005 and is funded through the French national rare diseases program, as well as industry grants. Their objectives are to organize and structure PID care in France, and improve knowledge and practices through education, training, and research and clinical projects.

The European Reference Network Board (ERN) Rare Immunological and Auto-Inflammatory Disorders (RITA) offers a clear, EU-endorsed framework to optimize cross border collaboration and care, along



with funding opportunities for research and clinical projects. IPOPI is now involved with this network to represent PID patient needs.

Mr. Prevot highlighted the important role of patient organizations in advocacy, citing IPOPI's role in advocating for transitional care for seniors with PID, the impact of Brexit on PID patients, and the importance of newborn screening as examples. They also play a large role in driving the implementation of the PID Principles of Care, and have an important part to play in education and awareness.



## Progress in Care in Canada

HAE – Dr. Stephen Betschel

[\(slide deck\)](#)

Dr. Betschel reviewed the current recommendations for comprehensive care of HAE in Canada, presented what has been achieved and areas that still need work, and proposed strategies to accomplish the yet unmet needs.

The Canadian HAE Guidelines were published in 2014. The guideline includes a recommendation for comprehensive care to be available to all patients with HAE with: a comprehensive team; access to specialized diagnostic testing and home treatment; a networked Patient Information System to facilitate product recalls and collect data on therapy outcomes and safety, and facilitate participation in clinical trials; access to clinical advances as they become available and to 24 hour support; standardized wallet cards; and tracking and intermittent audit of quality outcomes.

He noted that the prov/terr model of health care funding makes implementation of nationally uniform comprehensive care clinics challenging, but that the fundamentals of comprehensive care should still be equally accessible across the country, and that support should be provided by prov/terr governments to ensure that proper standards of care are being met. Treatment of HAE can be expensive, however inappropriate treatment of HAE may be even more costly.

Dr. Betschel shared about his own clinic at St. Michael's Hospital in Toronto, and cited studies showing the benefits, along with the Ontario Health Technology Advisory Committee Recommendation, for publicly-funded, home-based infusion treatment for patients with primary and secondary immunodeficiencies.

In conclusion he stated that comprehensive care is not new, and not unique to rare diseases. Treatments continue to evolve and move from hospital-based to home-based therapies, however there is no coordinated effort to track appropriate utilization of products and their efficacy. Comprehensive care clinics could manage this with appropriate support and would likely be cost effective. Bridging this gap will require the collaboration of health care providers, patients and advocates, and policy makers/payers.

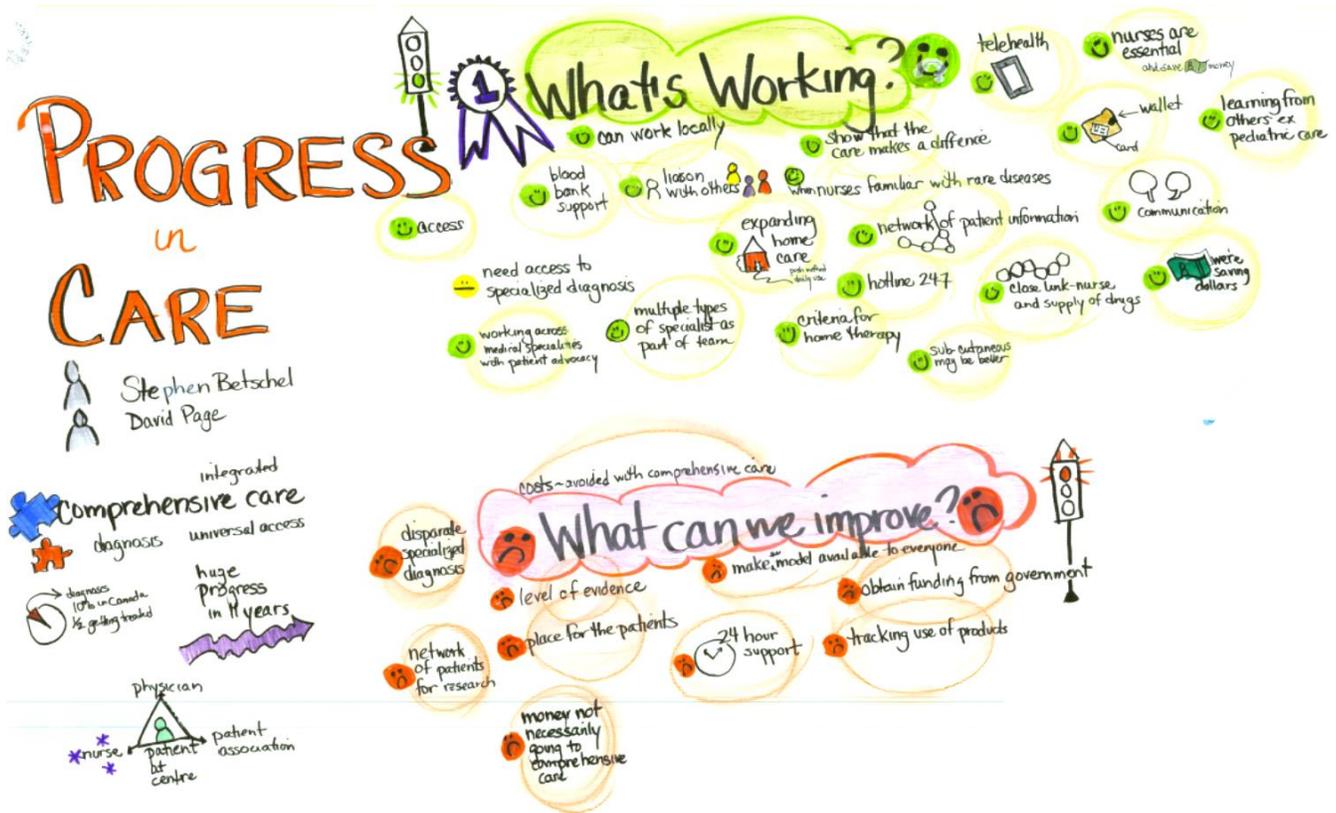
**PID – Dr. Jacques Hébert (presented by David Page)**  
[\*\(slide deck\)\*](#)

David Page presented Dr. Hébert's presentation in his absence. There are an estimated 15,000 PID patients in Canada, with only 10% diagnosed, and only 5% under treatment.

Dr. Hébert's clinic in Quebec is patient-centred, with physician specialists, a nurse coordinator, and the patient organization working together. They hold monthly clinics by 1 immunologist and one infectious disease specialist (Pediatric and adult) with easy access to ENT, GI, dermatology and hematology specialists. Infusion clinics are held 2 days/wk for IV infusions. There is also a 24/7 hot line for patient support.

They have a robust subcutaneous home infusion program, facilitated by the nurse coordinator, as this treatment is seen to be clinically effective, has an excellent safety profile, reduced adverse events, increased patient autonomy, and is less expensive for the patient and for society. He cited a CADTH report showing a savings of \$9M/yr if 75% of patients on IVIg switched to SCIg, or a savings of \$700/patient. There was also discussion of the pump vs push method, with the push method found to be simpler, faster, and less expensive.

Finally, the role of the patient organization was highlighted, carrying the vision of top quality care, an individualized approach, and keeping the quality of life of patients at the forefront.



### Panel Discussion

"What makes a compelling case that causes decision makers to champion funding for improved comprehensive care?"

Moderated by Mr. Ryan Clarke, Advocacy Solutions

Dr. Jacob Pendergrast, Hematologist, UHN Red Blood Cell Disorders Clinic

Ms. Sally Balmer, Blood Bank Manager, Toronto General Hospital

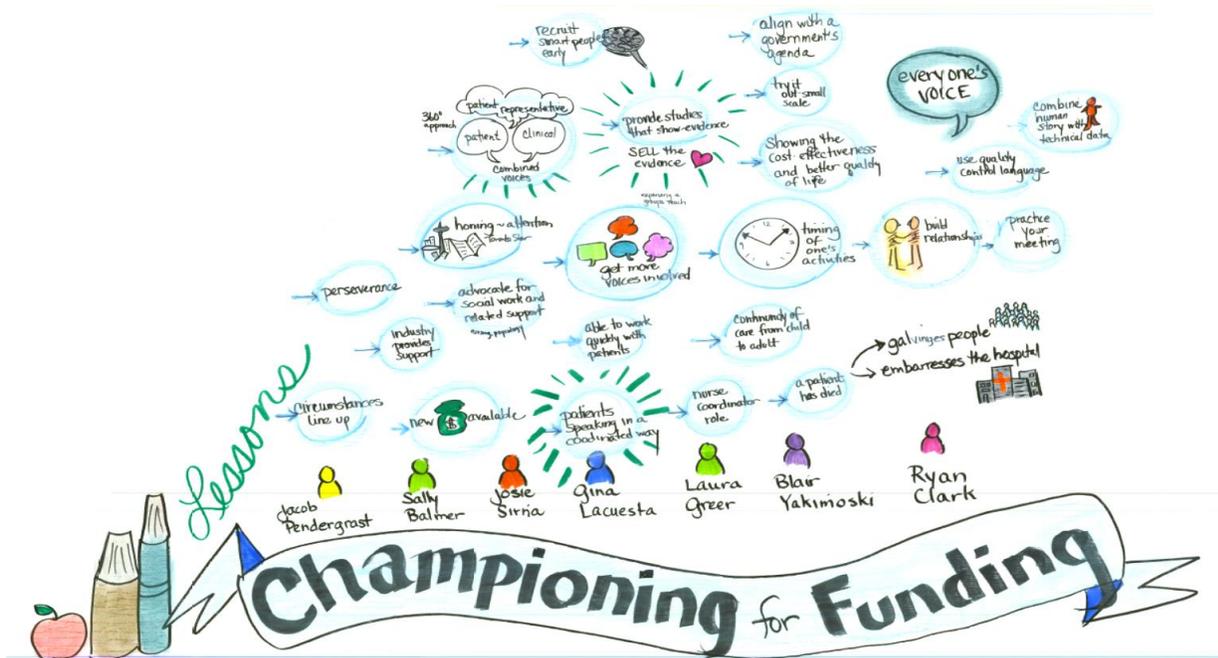
Ms. Josie Sirna, Thalassemia Foundation of Canada

Dr. Gina Lacuesta, Immunologist, Nova Scotia Health Authority

Ms. Laura Greer, Vice President, Hill+Knowlton Strategies

Mr. Blair Yakimoski, Manitoba MLA living with HAE

Our panelists shared valuable lessons learned from their own perspectives with advocating for comprehensive care as health care providers, hospital administrators, patient organization leaders, advocacy consultants, and politicians.



## Breakouts Sessions

Reports Back from Breakouts, Wrap-up & Conference Closing - Jennifer van Gennip

Breakout groups discussed what commitments were needed from each stakeholder group to move forward with comprehensive care, and reported back to the larger group.

The key takeaways were that we have a compelling case that includes evidence and patient stories to share with decision makers. What is needed is leadership from the patient groups, collaboration, and sustainable funding.